Therapeutic potential of Retinal Pigment Epithelial cell lines derived from hES cells for retinal degeneration.

Grant Award Details

Therapeutic potential of Retinal Pigment Epithelial cell lines derived from hES cells for retinal degeneration.

Grant Type: SEED Grant

Grant Number: RS1-00222

Investigator:

Name:	David Hinton
Institution:	University of Southern California
Type:	PI

Disease Focus: Aging, Vision Loss

Human Stem Cell Use: Embryonic Stem Cell

Award Value: $651,607

Status: Closed

Progress Reports

Reporting Period: Year 2

View Report

Grant Application Details

Application Title: Therapeutic potential of Retinal Pigment Epithelial cell lines derived from hES cells for retinal degeneration.

Public Abstract:

Retinal degeneration represents a group of blinding diseases that are increasingly impacting the health and well being of Californians. It is estimated that by 2020, over 450,000 Californians will suffer from vision loss or blindness due to the age-related macular degeneration (AMD), the most common cause of retinal degeneration diseases in the elderly. Furthermore, retinitis pigmentosa is the leading cause of inherited blindness in younger people. Currently there are no cures for these diseases. A layer of cells at the back of the eye called the retinal pigment epithelium (RPE), provide support, protection, and nutrition to the light sensitive retina, and cooperate with other retinal cells to maintain normal visual function. The dysfunction and/or loss of these RPE cells play a critical role in the development of the previously described blinding diseases. We suggest that effective treatment of retinal degeneration could be achieved by the proper replacement of damaged RPE and retinal cells with healthy ones. However, lack of the reasonable supply of healthy human eye cells hampers the application of this therapeutic approach. Recent advances in knowledge and technology of embryonic stem cells brings new hope for the development of cell replacement treatment. Embryonic stem (ES) cells are capable of unlimited self-replication and production of different cell types. RPE cells derived from human ES cells (hES-RPE) are a potentially unlimited resource for the cell replacement approach. We hypothesize that the dysfunction and/or loss of RPE can be replenished and restored through the transplantation of functionally polarized RPE monolayers derived from human embryonic stem cells, and this transplantation can cure the retinal degeneration diseases caused by RPE dysfunction.We propose to: 1. Derive RPE cells from human ES cells; 2. Establish and characterize the functionally mature or polarized monolayer of hES-RPE cells that will be suitable for transplantation; 3. Rescue the retinal degeneration phenotype through the transplantation of functionally mature or polarized monolayer of hES-RPE cells in animal models. Our goal is to determine the feasibility of treating the retinal degeneration diseases caused by RPE dysfunction through the transplantation of a monolayer of polarized hES-RPE cell sheet. The knowledge and technology from our research can be used to develop new treatments for human retinal degeneration diseases.

Statement of Benefit to California:

Age-related macular degeneration (AMD) is the leading cause of severe vision loss or blindness among the elderly, and currently no cure for this disorder exists. Based on the fact that California is the most populated state, and that the population continues to age, it is estimated that over 450,000 of Californians will suffer from AMD with severe vision impairment by 2020. Studies have shown that the devastating consequences of AMD include the progressive loss of independence and productivity, and increased risks of falls, fractures, and depression among diseased population. So this is not only a problem of the individual quality of life, but also an issue of increasing public health burden and concern. In this study we will test the feasibility of treating AMD and other retinal degenerative diseases through the transplantation of human embryonic stem cells that have been treated to differentiate into retinal pigment epithelial cells (RPE); the cells known to primarily degenerate or die in AMD. If our experimentation with RPE replacement therapy is successful in animal models, the knowledge and techniques can be quickly used to develop novel treatments for human diseases. Hundreds of thousands of Californians with AMD and other retinal degeneration diseases could benefit from our research for better quality of life with reduced morbidity. The California economy may significantly benefit from this work through potential reduced costs for health care, social welfare, and the loss of labor force.